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Abeona Therapeutics Receives Orphan Drug Designation in The European Union for ABO-102 Gene Therapy in Sanfilippo Syndrome Type A

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Abeona Therapeutics Inc. (NASDAQ: ABEO), a clinical- stage biopharmaceutical company focused on developing gene therapies for life-threatening rare diseases, announced today that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products has granted Orphan Drug Designation for Abeona's lead gene therapy program ABO-102 for the treatment of patients with Sanfilippo syndrome type A (MPS IIIA), a rare autosomal recessive disease that causes neurocognitive decline, speech loss, loss of mobility, and premature death in children.

"Receiving European Union (EU) orphan drug designation is an important milestone that delivers significant commercial benefits to our company as we advance our innovative portfolio of gene therapy products," stated Timothy J. Miller, Ph.D., President & CEO of Abeona Therapeutics Inc. "The benefits and incentives associated with these designations, including marketing exclusivity, are strategically important from a regulatory and commercial perspective and potentially value-creating for shareholders."

Abeona's MPS IIIA program, ABO-102, has previously been granted FDA Orphan Product Designation in the USA and received the Rare Pediatric Disease Designation as a pre-requisite part of the Priority Review Voucher (PRV) process.

About European Union (EU) Orphan Drug Designation: The European Commission grants orphan drug designation status to provide incentives to develop medicinal products to treat, prevent or diagnose diseases or conditions that affect no more than five in 10,000 persons in the European Union. The orphan drug designation provides Abeona with incentives and benefits in the EU, including reduced fees and protection from market competition once ABO-102 is approved for the treatment of MPS IIIA patients.

About ABO-102 (AAV-SGSH): ABO-102, the company's first-in-human, intravenously-administered AAV gene therapy, has been well tolerated through 30-day post injection in subjects injected with the low-dose (n=3). Encouraging signs of early biopotency have been observed in urinary and CSF GAG (glycosaminoglycan, specifically, heparan sulfate) measurements, as well as potential disease-modifying effects in the liver and spleen. The clinical study is supported by neurocognitive evaluations, biochemical assessments and MRI data generated in a 25-subject MPS III natural history study, also conducted at Nationwide Children's Hospital, where patients were evaluated for disease progression over one-year of

follow up assessments. ABO-102 is an adeno- associated viral (AAV)-based gene therapy for patients with MPS IIIA (Sanfilippo syndrome), that is delivered as a one-time intravenous injection. ABO-102 delivers a functioning version of the SGSH gene to cells of the central nervous system (CNS) and other organs with the goal of correcting the underlying deficits caused by the inborn genetic errors that are the cause the disease.

About Sanfilippo syndromes (or mucopolysaccharidosis)a group of four inherited genetic diseases each caused by a single gene defect, described as type A, B, C or D, which cause enzyme deficiencies that result in the abnormal accumulation of glycosaminoglycans (GAGs, or sugars) in body tissues. MPS III is a lysosomal storage disease, a group of rare inborn errors of metabolism resulting from deficiency in normal lysosomal function. The incidence of MPS III (all four types combined) is estimated to be 1 in 70,000 births. Mucopolysaccharides (GAGs) are long chains of sugar molecules used in building connective tissues in the body. There is a continuous process in the body of replacing used materials and breaking them down for disposal. Children with MPS III are missing an enzyme, which is essential in breaking down the used mucopolysaccharides called heparan sulfate. The partially broken down mucopolysaccharides remain stored in cells in the body causing progressive damage. In MPS III, the predominant symptoms occur due to accumulation of GAGs within the central nervous system (CNS), including the brain and spinal cord, and other tissues, which result in cognitive decline, motor dysfunction, and eventual death. Importantly, there is no cure for MPS III and treatments are largely supportive care.

About Abeona: Abeona Therapeutics Inc. is a clinical stage company developing gene and plasma-based therapies for life-threatening rare genetic diseases. Abeona's lead programs are ABO-102 (AAV-SGSH) and ABO- 101 (AAV-NAGLU), adeno-associated virus (AAV) based gene therapies for Sanfilippo syndrome (MPS IIIA and IIIB), respectively. Abeona is also developing EB-101 (gene-corrected skin grafts) for recessive dystrophic epidermolysis bullosa (RDEB), ABO-201 (AAV-CLN3) gene therapy for juvenile Batten disease (JNCL); ABO-202 (AAV-CLN1) gene therapy for treatment of infantile Batten disease (INCL), and ABO-301 (AAV-FANCC) for Fanconi anemia (FA) disorder using a novel CRISPR/Cas9-based gene editing approach to gene therapy for rare blood diseases. In addition, Abeona has a plasma-based protein therapy pipeline, including SDF Alpha™ (alpha-1 protease inhibitor) for inherited COPD, using our proprietary SDF™ (Salt Diafiltration) ethanol-free process. For more information, visit www.abeonatherapeutics.com.

This press release contains certain statements that are forward-looking within the meaning of Section 27a of the Securities Act of 1933, as amended, and that involve risks and uncertainties. These statements include, without limitation, our plans for continued development and internationalization of our clinical programs in Spain and Australia. These statements are subject to numerous risks and uncertainties, including but not limited to continued interest in our rare disease portfolio, our ability to enroll patients in clinical trials, the ability to successfully continue our clinical trials; the impact of competition; the ability to develop our products and technologies; the ability to achieve or obtain necessary regulatory approvals; the impact of changes in the financial markets and global economic conditions; and other risks as may be detailed from time to time in the Company's Annual Reports on Form 10-K and other reports filed by the Company with the Securities and Exchange Commission. The Company undertakes no obligations to make any revisions to the forward-looking statements contained in this release or to update them to reflect events or

circumstances occurring after the date of this release, whether as a result of new information, future developments or otherwise.

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